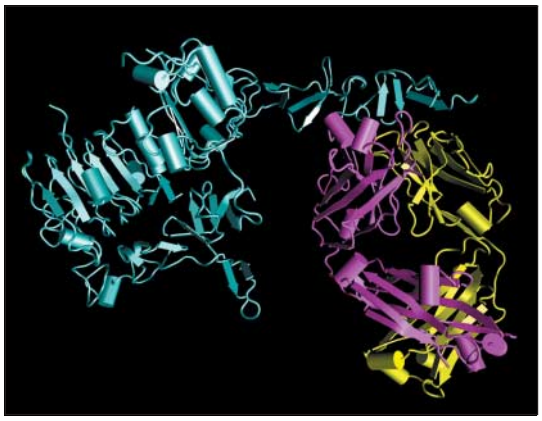
Medical paternalism and expensive unsubsidised drugs

Michael Jefford, Julian Savulescu, Jacqui Thomson, Penelope Schofield, Linda Mileshkin, Emilia Agalianos, John Zalcberg

When discussing treatment with patients, doctors may not mention newly licensed drugs that are not yet funded by healthcare schemes. Although their motives are good, the ethics are questionable

The development of new drugs can be a lengthy process, requiring initial laboratory and animal testing and then a course of clinical studies.[1] Clinical assessment involves phase I testing, which focuses on determination of side effects and an appropriate dose for later study; phase II studies, which assess efficacy in people with a particular condition; and phase III studies, which generally compare a new, experimental treatment with an existing standard treatment. Once a therapeutic benefit has been shown, the drug company can apply for approval from the relevant regulatory body. An extensive review follows, which may take many months or years. If the drug is approved, further delays may occur before funding arrangements permit the drug to be widely available. We consider some of the ethical dilemmas surrounding this process, using the example of drugs to treat people with cancer.



DR TIM EVANS/SPL

Molecular model of Herceptin: delays in funding expensive drugs cause dilemmas for doctors

Unsubsidised, expensive drugs

In Australia, the cost of most prescription medicines is subsidised by the government through the pharmaceutical benefits scheme. New drugs that have been approved by the Therapeutic Goods Administration are assessed for inclusion in the scheme by the Pharmaceutical Benefits Advisory Committee, an independent expert committee consisting of medical practitioners and pharmacists. The committee considers several issues before recommending a drug, including efficacy, safety, quality of life benefits, and cost effectiveness. The committee may apply restrictions on how drugs can be prescribed. Long delays can occur between the Therapeutic Goods Administration approving a new drug and listing on the pharmaceutical benefits scheme.

Similar delays between approval and subsidisation exists in other countries. In the United Kingdom, the National Institute for Health and Clinical Excellence (NICE) has been accused of considerable delays in making drugs available through the NHS. The cancer charity, CancerBACUP, has recently compiled a "dossier of delay," outlining major delays in approval of new anticancer drugs.[2] Some drugs that have been shown to produce important survival gains in large clinical trials may not be recommended by NICE for several years, making access difficult for many patients. Similar delays also occur in other European countries as well as in Canada and New Zealand.

Practice of discussing unfunded drugs

Several drugs for treating cancer (chemotherapy and biological agents) have recently been studied in large phase III clinical trials and shown to be more effective than existing treatments. However, as the new drugs are not on the pharmaceutical benefits scheme list patients must pay their full cost unless they are provided by a public hospital. Costs can be considerable, commonly $A1000 a week (£420, €600, $730). For many people such costs would represent a major financial burden or be totally prohibitive.

Discussing use of unsubsidised drugs with patients raises several potential dilemmas for clinicians. Among these, is it reasonable to ask a patient to finance the full cost of their treatment when it is not provided by government? Would it be unethical not to raise the option of treatment with an unsubsidised drug? And how should the oncologist discuss the option of an unsubsidised drug with a patient or their family?

We examined the opinions and practices of Australian medical oncologists regarding unsubsidised drugs. We sent a survey to all 274 members of the Medical Oncology Group of Australia that described three clinical scenarios in which treatment with a hypothetical unsubsidised drug was associated with a significant, objective benefit that had been confirmed in large clinical trials. The examples were based on treatments that had recently been reported in the

Division of Haematology and Medical Oncology, Peter MacCallum Cancer Centre, Melbourne, Locked Bag 1, A'Beckett Street, Victoria 8006, Australia
Michael Jefford *medical oncologist*
Jacqui Thomson *medical oncologist*
Penelope Schofield *behavioural scientist*
Linda Mileshkin *medical oncologist*
Emilia Agalianos *research assistant*
John Zalcberg *medical oncologist*
Oxford Uehiro Centre for Practical Ethics, University of Oxford, Oxford
Julian Savulescu *ethicist*
Correspondence to: M Jefford Michael.Jefford@petermac.org

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medical literature (trastuzumab for women with breast cancer, imatinib for treatment of gastrointestinal stromal tumours, and gemcitabine based treatment for people with advanced bladder cancer).[3–5] At the time the drugs were not available through the pharmaceutical benefit system. Although each is now available, it took many months or years before they were listed.

We had a 78% response rate to the survey (38 were ineligible; of the remaining 236, 184 returned questionnaires). Most oncologists indicated that they would discuss the new drug with the patient if it were subsidised (72%-94% of eligible responses (128-169), depending on the scenario). However, if the drugs were not subsidised, oncologists were considerably less likely to discuss the treatment option (28%-41% (50-72), depending on the scenario). Most commonly cited reasons for not discussing the drug were "the knowledge that they could not obtain this new drug would be too distressing for the patient and their family" and "I would feel bad mentioning a medication that the patient probably cannot afford."

Our findings suggest that oncologists are concerned about the potential psychological and emotional effect that these discussions might have on patients and their families. The findings also indicate that these discussions are stressful for practitioners. Nevertheless, we query whether this practice is necessarily in the patient's best interests and whether such an approach is inappropriately paternalistic.

Ethics of discussing unfunded drugs

Three principles effectively govern medical ethics: beneficence (promoting the best interests of patients), respect for autonomy, and distributive justice (distributing limited resources fairly).[6] A deep and unresolved ethical issue remains about when doctors should inform patients of options, including the possibility of new drugs, when evidence concerning possible benefit or risk is weak or non-existent, regardless of issues around distributive justice. Doctors should not recommend harmful interventions. But how strong should the evidence for an option be for it to be reasonable? What balance of benefits over risks should there be?

Above, we have considered drugs that seem to be reasonable options if cost were not an issue, in the context where cost is indeed an issue. When should these options be discussed? The evidential test we suggest is: would a reasonable doctor offer or would this patient (given their individual values) reasonably desire this drug or treatment if it were free?

The principle of respect for personal autonomy strongly supports providing patients with any information that might be relevant to a treatment decision based on their own particular values and attitudes to risk. The Australian High Court has endorsed this position.[7] Patients need to understand all relevant facts to make autonomous decisions about their health. This includes information about medical treatments or procedures that they may find of value and might be prepared to fund from their own resources. It may be viewed as paternalistic to deny patients information which may be of value to them and paternalistic to believe that doctors can predict that patients would either not want this information or be too distressed by a potential inability to pay for the drug. Moreover, it is difficult for doctors to know the financial capacity of individual patients or their relatives. Whether patients want to be informed about unsubsidised drugs to treat cancer has not been specifically studied, although the literature generally suggests that most patients want as much information as possible[8] and may seek information from many sources, including the internet.[9]

Most oncologists in our study seem to be motivated by beneficence and non-maleficence. Interestingly, in each of the three scenarios, the drug was plausibly in the patient's medical interests, offering a potential advantage in either survival or quality of life. The reason that the drug might not be in a patient's interest was cost, and the attendant emotional and other effects that financial burden may impose on the patient's life—that is, it might not be in the patient's global interests. However, it is inappropriate for doctors to make an evaluation of what is in a person's overall or global interests. Not only does withholding information about unsubsidised drugs fail to respect autonomy, it may not be in the patient's interests.

Distributive justice might require that drugs for which there is evidence of only minimal effect are not publicly funded, but it cannot prohibit people accessing treatment they desire using their own funds unless it is unsafe. Doctors should be committed to the individual patient's interests and autonomy rather than to their own conception of social ideals, such as equality.

We therefore believe there are no good reasons for withholding information about new unsubsidised drugs when reasonable evidence is available on safety and efficacy. Money is an instrumental good, merely a means to obtain other goods such as longevity and wellbeing. Patients may trade various components of their wellbeing to maximise overall welfare. Money is a means to promote wellbeing, and it makes sense to trade large amounts of it for health, just as it makes sense to give up other things in one's life to be healthy. A patient preference to do this is evidenced by the large amount of money many patients spend on unproved treatments in the belief that they might benefit health.[10 11] Decisions about how to maximise overall wellbeing should be the patient's own; hence, the decision of how much to spend on healthcare that is not provided by government should be the patient's own.

Patients may, of course, decide to forsake their health and expensive medical options, perhaps for the benefit of their families or others. In such cases, offering tempting expensive drugs may compromise rather than promote their autonomy. But withholding information on the basis of what a patient would want is a dangerous medical path to unjustified paternalism. It is difficult for patients, their families, and doctors to accurately predict what a competent patient would want in the face of serious or lethal illness. In such complex cases, the default position of medical consultation should be to inform patients non-directively of reasonable options, without attempting to persuade them to take unsubsidised drugs. If patients have clearly and relevantly stated in advance that they do not want certain options, including unsubsidised drugs, knowing the consequences, that would be a reason not to inform them, though this is rarely the case.

Access to drugs in development

Regulatory bodies exist to ensure that drugs are not made available until they have been shown to be safe and effective. Because the process of developing new drugs can take several years, some people argue that regulatory agencies also deny access to potentially useful drugs.[12] This could also be seen as a form of paternalism.

Access to drugs in development is not a simple case of allowing people to make their own choices, since patients harmed by unsafe interventions require medical care. Ensuring that patients are adequately informed about the potential risks and benefits of investigational treatments certainly presents major challenges, although it may be considered analogous to participation in early phase clinical trials. And when is it necessary for clinicians to disclose to their patients that a drug may have potential benefit? Is it paternalistic to prevent patients from accessing drugs very early in their development? Could it be paternalistic even before phase I toxicity studies have been completed? People with life threatening illnesses may be vulnerable, but, again, this is not an adequate reason to deny access to potentially useful treatments.

Cost of new drugs

A further ethical issue concerns the cost of publicly funding newer, expensive treatments. Schrag has recently reflected on the effect of new drugs for advanced colorectal cancer.[13] Although these drugs have nearly doubled the median survival over the past decade, the drug cost has risen by "a staggering 340-fold."[13] Other authors have recently queried whether society can afford to pay for newer biological drugs.[14] If society is not prepared to provide new drugs in a timely manner, should individuals not be allowed the opportunity to consider purchasing them in an effort to improve their health? Unfortunately, evidence shows that as patients are asked to pay for their drugs, even in the form of co-payments (which might be a small percentage of the total cost), those less able to afford these costs suffer worse health outcomes.[15–17] This is an urgent question of distributive justice that faces not just new drugs but many forms of modern technological care. The gap between what we can do and what we can afford will continue to grow, at a personal and community level.

Conclusions

The issue of accessing new expensive drugs has no easy solutions. Nevertheless, at the least it seems unreasonable to withhold information from a patient about any potentially beneficial treatment because of concerns about capacity to pay, even if these discussions may be difficult and cause distress for some patients. We believe that it is generally unethical and paternalistic to withhold such information. Additional challenges concern access to promising drugs early in their clinical evaluation and whether or how to publicly fund high cost drugs. Society at large will need to engage in debate about how much responsibility the state should take for individual health and wellbeing and about how limited healthcare funds should be allocated.

Summary points

Long delays often occur between licensing of a drug and its availability through publicly funded health schemes

Some unfunded expensive drugs may be preferable treatments to funded drugs

A large proportion of oncologists would not discuss an expensive drug with a patient if it were not subsidised

Doctors should inform patients of unsubsidised drugs if they judge that patients would want the treatment if it were free

Many new drugs are very expensive, and society should discuss whether these drugs should be publicly funded

Contributors and sources: JZ, MJ, JT, and LM are all medical oncologists with considerable clinical experience, including dealing with discussions around high cost drugs. The idea for surveying medical oncologists arose from conversations about this clinical encounter. PS and MJ have experience in the design of postal questionnaires. JS has researched and published widely on medical ethics. MJ wrote the initial draft of the paper. JS wrote most of the ethical analysis. JT and JZ developed the original idea and the initial study proposal. All authors developed the study protocol. EA coordinated the data collection. PS performed the data analysis. All authors reviewed this manuscript, and MJ is the guarantor.

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